
CIRM-Funded Therapy for Disease that Attacks Vision Gets Go Ahead for Clinical Trial

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12th CIRM-funded clinical trial to gain approval

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San Francisco, CA – A novel stem cell therapy for a disease that slowly destroys a person's vision has been given approval to start a clinical trial by the Food and Drug Administration (FDA).

Dr. Henry Klassen and his team at the University of California, Irvine (UCI) have developed the therapy to treat retinitis pigmentosa (RP), which erodes the cells in the retina, the light sensitive area in the back of the eye that is crucial for vision. The clinical trial will involve enrolling up to 16 patients who will get a single injection in the eye of stem cells known as retinal progenitors. It's hoped these will help protect photoreceptors that are not yet damaged by the disease, and even replace those that are already lost.

CIRM, California's stem cell agency, has invested almost \$20 million in helping bring the therapy through early research and into the clinical trial.

"One of the goals of the agency is to provide the support that promising therapies need to progress and ultimately to get into clinical trials in patients," says Jonathan Thomas, Ph.D., J.D., Chair of the agency's governing Board. "RP affects about 1.5 million people worldwide and is the leading cause of inherited blindness in the developed world. Having an effective treatment for it would transform people's lives in extraordinary ways."

The goal of this first phase clinical trial is to test the safety of the therapy but researchers will also be looking to see if it has any effect on vision and the overall function of the eye.

"This milestone is a very important one for our project," said Klassen. "It signals a turning point, marking the beginning of the clinical phase of development, and we are all very excited about this project."

This is the 12th project funded by the stem cell agency to get approval from the FDA to carry out a clinical trial. Other recent approvals include therapies for HIV/AIDS, macular degeneration (the leading cause of vision loss in the elderly) and beta thalassemia.

Klassen says without the agency's support he doubts his project would have made it this far: "CIRM has played a critical and essential role in this project. While the funding is extremely important, CIRM also tutors and guides its grantees in the many aspects of translational development at every step of the way, and this accelerates during the later pre-clinical phase where much is at stake."

The trial, which will begin pending approval from the UCI Institutional Review Board, will be conducted the Gavin Herbert Eye Institute on the UCI campus, and at the UC Irvine Medical Center in Orange.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and to act with a sense of urgency commensurate with that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and over 280 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of medicine closer to reality.

About the University of California, Irvine

Currently celebrating its 50th anniversary, UCI is the youngest member of the prestigious Association of American Universities. The campus has produced three Nobel laureates and is known for its academic achievement, premier research, innovation and anteater mascot. Led by Chancellor Howard Gillman, UCI has more than 30,000 students and offers 192 degree programs. It's located in one of the world's safest and most economically vibrant communities and is Orange County's second-largest employer, contributing \$4.8 billion annually to the local economy. For more on UCI, visit www.uci.edu

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